



## INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

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<b>(21) International Application Number:</b> PCT/US96/14423 <b>(22) International Filing Date:</b> 6 September 1996 (06.09.96) <b>(30) Priority Data:</b> 60/003,470 8 September 1995 (08.09.95) US <b>(60) Parent Application or Grant</b> <b>(63) Related by Continuation</b> US 60/003,470 (CIP) Filed on 8 September 1995 (08.09.95) <b>(71) Applicant (for all designated States except US):</b> GENZYME CORPORATION [US/US]; One Mountain Road, Framingham, MA 01701 (US). <b>(72) Inventors; and</b> <b>(75) Inventors/Applicants (for US only):</b> WADSWORTH, Samuel, C. [US/US]; 10 Straw Hollow Lane, Shrewsbury, MA 01545 (US). VINCENT, Karen [US/US]; 337 Park Avenue, Arlington, MA 02174 (US). PIRAJNO, Susan [US/US]; Apartment 411B, 1622 Worcester Road, Framingham, MA 01701 (US). KYOSTIO, Sirkka [FI/US]; 61 Winter Street, Framingham, MA 01701 (US).	<b>(74) Agent:</b> DUGAN, Deborah, A.; Genzyme Corporation, One Mountain Road, Framingham, MA 01701 (US). <b>(81) Designated States:</b> AU, CA, JP, US, European patent (AT, BE, CH, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE). <b>Published</b> <i>Without international search report and to be republished upon receipt of that report.</i>	
<b>(54) Title:</b> IMPROVED AAV VECTORS FOR GENE THERAPY		
<b>(57) Abstract</b> <p>The present invention is directed to methods for generating high titer, contaminant free, recombinant AAV vectors, methods and genetic constructs for producing AAV recombinant vectors conveniently and in large quantities, methods for the delivery of all essential viral proteins required in <i>trans</i> for high yields of recombinant AAV, recombinant AAV vectors for use in gene therapy, novel packaging cell lines which obviate the need for cotransfection of vector and helper plasmids, helper plasmids and vector plasmid backbone constructs, a reporter assay for determining AAV vector yield. Further provided are recombinant AAV vectors in a pharmaceutically acceptable carrier, methods of delivering a transgene of interest to a cell, compositions and methods for delivering a DNA sequence encoding a desired polypeptide to a cell, and transgenic non-human mammals that express a human chromosome 19 AAV integration locus.</p>		

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